

ADVISORY COMMITTEE BRIEFING MATERIALS: AVAILABLE FOR PUBLIC RELEASE

**ERRATA FOR ADVISORY COMMITTEE BRIEFING DOCUMENT**

**DRISAPERSEN**

**NDA 206031**

**PERIPHERAL AND CENTRAL NERVOUS SYSTEM DRUGS**

**ADVISORY COMMITTEE**

24 November 2015



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This errata provides minor corrections to one Table contained within the briefing document to support the upcoming Advisory Committee meeting on 24 November 2015. This errata is intended to provide transparency of these minor corrections and does not change the data in the table or affect the conclusions within the briefing document.

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**PAGE 108, TABLE 4.7.2.2.3**

**Was:**

**Table 4.7.2.2.1: Adjusted Mean Change from Baseline in 6MWD at Week 48 Adjusted for Age, Baseline 6MWD, and Baseline Rise-from-the-Floor Time in Study 044 (ITT Populations)**

	<b>Placebo (n=61)</b>	<b>Drisapersen 6 mg/kg/wk (n=125)</b>
<b>Baseline 6MWD, n</b>	9	17
Mean $\pm$ SD, meters	361.8 $\pm$ 85.4	363.0 $\pm$ 114.3
<b>Week 48, n</b>	<b>9</b>	<b>16</b>
Mean change $\pm$ SE, meters	<b>-108.6 <math>\pm</math> 24.8</b>	<b>-46.9 <math>\pm</math> 18.4</b>
Mean difference vs. placebo, meters	–	<b>61.7</b>
(95% CI)	–	<b>(-2.2, 125.6)</b>
p-value	–	<b>0.058</b>

6MWD, 6-minute walking distance in meters; CI, confidence interval; MMRM, mixed model repeated measures; ITT, intent-to-treat; SD, standard deviation; SE, standard error.

All mean changes are adjusted.

MMRM model included terms for treatment, visit, treatment by visit, age, age by visit, country grouping, baseline 6MWD, and baseline 6MWD by visit.

**Is amended to:**

**Table 4.7.2.2.3: Adjusted Mean Change from Baseline in 6MWD at Week 48 of Patients in Study 044 from Centers that Previously Participated in Study 117**

	<b>Placebo (n=61)</b>	<b>Drisapersen 6 mg/kg/wk (n=125)</b>
<b>Baseline 6MWD, n</b>	9	17
Mean $\pm$ SD, meters	361.8 $\pm$ 85.4	363.0 $\pm$ 114.3
<b>Week 48, n</b>	<b>9</b>	<b>16</b>
Mean change $\pm$ SE, meters	<b>-108.6 <math>\pm</math> 24.8</b>	<b>-46.9 <math>\pm</math> 18.4</b>
Mean difference vs. placebo, meters	–	<b>61.7</b>
(95% CI)	–	<b>(-2.2, 125.6)</b>
p-value	–	<b>0.058</b>

6MWD, 6-minute walking distance in meters; CI, confidence interval; MMRM, mixed model repeated measures; SD, standard deviation; SE, standard error.

All mean changes are adjusted.

MMRM model included terms for treatment, visit, treatment by visit, country grouping, baseline 6MWD, and baseline 6MWD by visit.

Rationale: Changes were made to table title and footnotes to clarify that analysis was performed on the group of patients in Study 044 from centers that previously participated in Study 117 and that the MMRM model included terms for treatment, visit, treatment by visit, country grouping, baseline 6MWD, and baseline 6MWD by visit, and did not include terms for Age and Baseline Rise-from-the-Floor Time.